

burden. Improving the control of PD motor symptoms on patients may contribute to prevent anxiety and HRQoL deterioration in caregivers.

PND4

A COMPREHENSIVE LITERATURE REVIEW OF THE BURDEN OF GAUCHER DISEASE

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OBJECTIVES: Gaucher disease (GD) is an inherited, rare, lysosomal storage disorder caused by a genetic deficiency of glucocerebrosidase. The result is the accumulation of the substrate, glucosylceramide, in the lysosomes of macrophage cells in the liver, spleen, bones, lungs, and other vital tissues. Three subtypes of Gaucher disease are recognized: type 1 (GD1, non-neuropathic), type 2 (GD2, acute neuropathic), and type 3 (GD3, subacute/chronic neuropathic). Clinical manifestations of the disease are multisystemic, clinically heterogeneous and require lifelong management. **METHODS:** To better understand the burden of GD, a comprehensive review of the published literature was conducted. MEDLINE, EMBASE, CENTRAL and “grey” literature sources published in English between January 1990 and February 2013 were searched for relevant publications. **RESULTS:** A total of 97 publications focusing on the epidemiologic, clinical, and socioeconomic burden of GD, treatment options and guidelines were summarized. The standardized incidence and prevalence of GD in the general population varies from 0.30 to 5.80 per 100,000 and 0.33 to 1.75 per 100,000, respectively, and GD1 is the predominant type in most regions. The risk of mortality is highest in GD patients younger than age 5 years and generally increased after age 55; the life expectancy is lower than the general population. Common manifestations of GD such as anemia, thrombocytopenia, splenomegaly, hepatomegaly and bone disease lead to a decreased quality of life. Reported GD comorbidities include Parkinson’s disease and cancer. Current treatment options consist of enzyme replacement therapy (ERT, standard of care) and substrate reduction therapy (SRT). ERT is the standard of care, though unmet needs still exist, especially for GD2 and GD3. **CONCLUSIONS:** GD is a rare, chronic disease associated with significant burden to patients and caregivers. While ERT is an effective and well-established treatment for GD patients, several unmet needs exist and further research is needed in this area.

PND5

RESTLESS LEG SYNDROME DETECTION IN HEMODIALYSIS

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OBJECTIVES: Restless leg syndrome (RLS) is a condition with possibly high prevalence in hemodialysis (6–60% according to the literature), and a specific treatment available. Thus it’s important to identify it among other conditions present in this population that might confound diagnosis (such as peripheral vascular disease or neuropathies). An approach based on a self-completed screening test will be assessed in this study, along with an estimation of RLS prevalence in hemodialysis. **METHODS:** Patients from two hemodialysis units answered a RLS screening test. Those with a positive screening completed the International Restless Legs Syndrome Study Group Rating Scale (IRLS) that assesses symptom severity. A neurophysiologist performed a clinical interview to confirm the diagnosis, including a supervised administration of the IRLS. **RESULTS:** 164 patients were recruited. Mean age was 65.7 years (range 33–87; P<25–75: 55.5–77.5), 67% were male and mean time in dialysis was 64.16 months. Self-completed screening test identified 69 possible cases of RLS (42.07%). 44 (26.8%) patients had RLS symptoms according to the self-completed IRLS, and 79% of them were classified as having moderate to severe RLS symptoms. The clinician confirmed just 22 of those cases (13.4% of the total sample), with a demographic profile similar to the sample. The screening test had in this sample a sensitivity of 100%, specificity 66.43% and positive predictive value 31.88%. **CONCLUSIONS:** This study found RLS is a relatively common condition in hemodialysis patients. The screening test showed a high sensitivity to detect RLS, but very low specificity, so the confirmation of an expert neurologist or neurophysiologist is necessary.

PND6

CEREBROSPINAL FLUID F¹⁸-AMYLOID1-42 LEVELS IN THE DIFFERENTIAL DIAGNOSIS OF ALZHEIMER’S DISEASE - SYSTEMATIC REVIEW AND META-ANALYSIS

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OBJECTIVES: The purpose of this study was to carry out systematic review of the literature and meta-analysis to evaluate the diagnostic utility of cerebrospinal fluid (CSF) levels of the 42 amino acid form of amyloid- β (Ab₁₋₄₂) as a biomarker for differentiating Alzheimer’s disease (AD) from non-AD dementia. **METHODS:** Design - Systematic literature review was used to evaluate the effectiveness of the Ab for the diagnosis of Alzheimer’s disease. The Scottish Intercollegiate Guidelines Network (SIGN) tool was used by two evaluators to evaluate independently the quality of the 15 studies. Data sources - The literature review covered from October 27, 1946, to October 22, 2013, and searched eight domestic databases including Korea Med and international databases including Ovid-MEDLINE, EMBASE, and Cochrane Library. Eligibility criteria for selecting studies - Primary criteria for inclusion were valid studies on (i) patients with mild cognitive impairment with confirmed or suspected AD and non-AD dementia, and (ii) assessment of Ab₁₋₄₂ levels using appropriate comparative tests. **RESULTS:** A total of 15 studies (15 diagnostic evaluation studies) were identified in which levels of CSF Ab₁₋₄₂ were assessed. Meta-analysis was performed on nine robust studies that compared confirmed AD with healthy individuals ($n = 1587$), 10 studies that compared AD with non-AD dementias ($n = 860$), and four studies that compared a-MCI (amnestic mild cognitive impairment) with na-MCI (non-amnestic mild cognitive impairment) subjects ($n = 857$). Overall, Ab₁₋₄₂ levels were reduced in CSF from AD patients versus healthy controls or non-

AD dementia. The effectiveness of this test was evaluated for diagnostic accuracy. Diagnostic accuracy for identifying AD by ELISA was high (pooled sensitivity, 0.772 (95% CI 0.747–0.796); pooled specificity, 0.732 (95% CI 0.699–0.762). **CONCLUSIONS:** Reduced CSF Ab₁₋₄₂ levels are of potential utility in the differential diagnosis of AD versus non-AD dementias and healthy controls.

PND7

PREVALENCE OF CYSTIC FIBROSIS AMONG THE U.S. NATIONAL MEDICAID POPULATION

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OBJECTIVES: Cystic fibrosis (CF) prevalence according to U. S. geographic region as well as patient age, gender and race was examined in the U. S. Medicaid population for patients younger than age 45. **METHODS:** Patients ≤ 45 years from the Medicaid fee-for-service (FFS) population (2008–2009) were identified using International Classification of Disease 9th Revision Clinical Modification (ICD-9-CM) diagnosis code 277.0x. Patients were required to have continuous Medicaid FFS enrollment in both years and no evidence of managed care enrollment. CF prevalence was stratified by U. S. region, state, age group, gender and race, and was measured by number and percentage of patients in each category. **RESULTS:** A total of 2,142 patients were diagnosed with CF among the Medicaid FFS population under age 45 years in 2008 and 2009. Prevalence was the highest (0.17%) for patients under age 17 years, followed by those age 18–35 (0.14%), and 36–45 years (0.06%). However, some states had the highest CF prevalence in the 18–35 age range (Colorado: 0.92%; North Dakota: 0.50%; Kentucky: 0.54%). CF prevalence by race was also examined with the following results: White (0.17%), Hispanic (0.10%), Asian (0.07%), Black (0.06%) and Native American (0.03%). Male patients had a relatively higher prevalence than female patients (0.14% vs. 0.12%). The highest CF prevalence was observed in Colorado (0.47%), followed by Maryland (0.46%), North Dakota (0.31%), Ohio (0.28%) and Pennsylvania (0.27%). Patients residing in the Midwest U. S. region had the highest prevalence rate (0.15%), compared to the South (0.15%), Northeast (0.12%) and West (0.04%) regions. **CONCLUSIONS:** CF prevalence was the highest in patients age <17 years nationwide, however, certain states showed the highest prevalence among patients age 18 to 35. White and male patients residing in the Midwest U. S. region were found to be at higher risk of a CF diagnosis.

PND8

RISK OF RELAPSE AMONG PROPENSITY SCORE MATCHED MULTIPLE SCLEROSIS PATIENTS RECEIVING NATALIZUMAB OR PLATFORM THERAPY IN THE US

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OBJECTIVES: To examine claims-based relapse rates and time to relapse among multiple sclerosis (MS) patients treated with natalizumab or propensity score matched patients treated with platform therapy (interferon beta/glatiramer acetate) in the US. **METHODS:** The Truven Health MarketScan Research Databases were used to identify adults with a MS (ICD-9-CM code 340) diagnosis treated with natalizumab or platform therapy; the first claim between January 1, 2009 and April 1, 2012 was the index. Patients had to have one year continuous enrollment pre- and post-index and remain on index therapy for 12 months. Patients were excluded if they used a non-index therapy in the pre-index. Natalizumab and platform patients were propensity score matched using nearest neighbor matching on demographic characteristics, selected comorbid conditions and medications, MS severity (using an adaptation of Kurtzke’s Functional System), pre-index relapse and pre-index expenditures. MS-relapse was defined as MS-related inpatient (IP) admission, IV or oral corticosteroid use. Cox Proportional Hazard models were used to evaluate time to relapse, controlling for demographic and pre-index clinical characteristics. **RESULTS:** A total of 897 natalizumab patients met the study criteria, 882 of which were 1:1 matched to 882 platform therapy patients (mean age 45 years, 70% female) with a standardized difference <10 on all matching measures. Compared to platform patients, natalizumab patients were significantly less likely to have MS-relapse post-index (26.5% vs. 35.5%, $p < 0.001$), with lower post-index rates of MS-related IP admissions (1.0% vs. 2.6%), IV-corticosteroid use (15.6% vs. 19.0%) and oral corticosteroid use (15.4% vs. 23.1%) (all $p < 0.001$). Natalizumab patients also had 25 more relapse-free days (308 vs. 283 days, $p < 0.001$). Post-index MS-relapse risk was lower for natalizumab patients (HR=0.69, $p < 0.001$) after controlling for baseline characteristics. **CONCLUSIONS:** Natalizumab was associated with a significantly lower risk and rate of MS-relapse and had longer time to a MS-relapse compared to platform therapy.

PND9

IMAGE-GUIDED NAVIGATION SYSTEMS (IGNS) IMPROVE ACCURACY OF CATHETER PLACEMENT IN SHUNTED HYDROCEPHALUS PATIENTS

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BACKGROUND: The most common surgical complication associated with shunt placement in Hydrocephalus patients is obstruction causing shunt malfunction. The primary cause of obstruction is incorrect placement of the catheter tip, most notably in the choroid plexus. **OBJECTIVES:** To investigate the clinical and economic value of IGNS use in the accurate placement of catheters in Hydrocephalus patients. **METHODS:** A search of the Embase and PubMed electronic databases was conducted to identify studies evaluating the accuracy, effectiveness, quality-of-life (QoL) and economic aspects of IGNS in patients with Hydrocephalus. No language restrictions were applied. **RESULTS:** We conducted a meta-analysis of studies reporting accuracy of ventricular catheter placement in patients with hydrocephalus undergoing shunt placement with stereotactic IGNS versus freehand technique. The definition of accurate catheter placement was similar in all studies. The meta-analysis showed the odds of achieving an accurate catheter placement for surgeons who utilize IGNS was almost 6 times higher (odds ratio 5.55, 95% CI [2.84, 10.85], $P < 0.00001$) than surgeons who used freehand placement techniques. Furthermore, accurate place-